

# RNA Therapeutics Landscape<sup>(1)</sup>

## Highest Stage of Development

| Approach                                    | Discovery / Preclinical                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                        | Phase 1 & 1/2                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                           | Phase 2                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                 | Phase 3 / Reg                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                           | Approved                                                                                                                                                                                                                                                                                                                                        |
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| ASOs / PMOs                                 |                                                                                                                                                                                                                                                                      |       |                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                 |       |     |
| RNAi                                        |                |                                                                                                                                                                             |               |                                                                                          |                                                                                                                                                                           |
| mRNA / RNA Aptamers                         |                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                |       |                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                          |                                                                                                                                                                                                                                                                |                                                                                        |
| Other RNA Molecules (tRNA, circRNA, lncRNA) |                                                                                  |                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                         |                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                         |                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                         |                                                                                                                                                                                                                                                                                                                                                 |
| RNA-Targeted Small Molecules                |                                                                                                                                                                                                                                             |                                                                                                                                                                                                                                                                                                                                                                                                                                    |                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                         |                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                         |                                                                                                                                                                       |
| RNA Editing & Modulation                    |                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                          |                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                         |                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                         |                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                         |                                                                                                                                                                                                                                                                                                                                                 |

Andrew Pannu  @andrewpannu

(1) As of 3/28/2023, not exhaustive – both public & private companies only